Challenges in preparing HTA submission dossier

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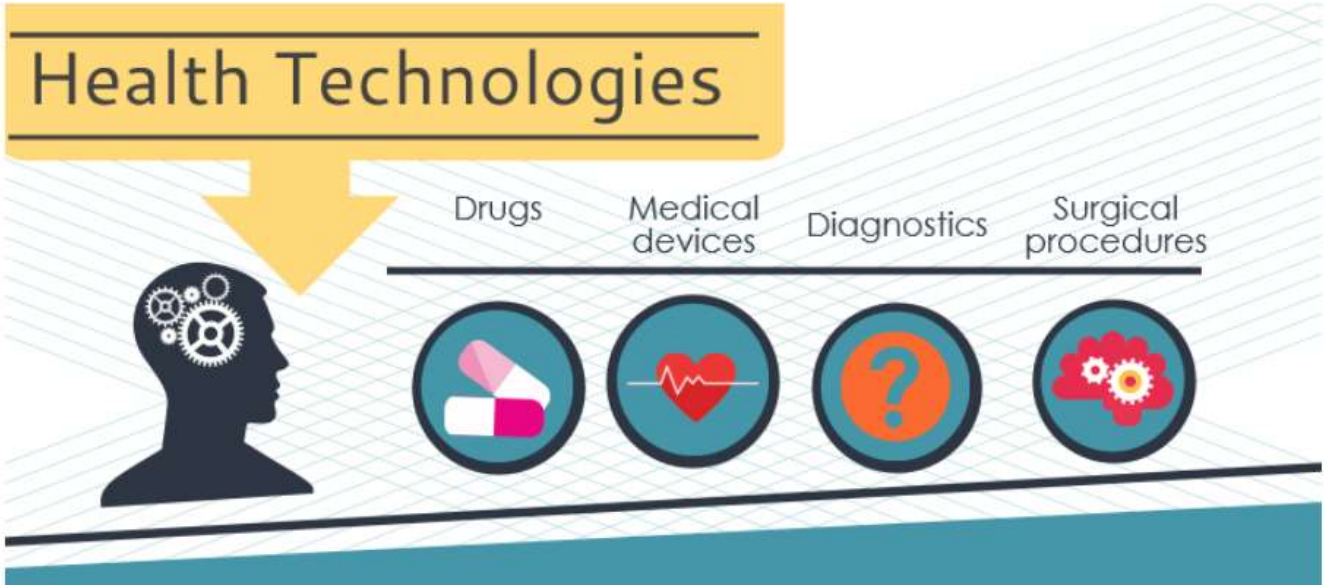
Health Technology Assessment

- Health technology assessment (HTA) is a multidisciplinary research process that collects and summarises information about a health technology.
- The information can cover a range of fields, including clinical effectiveness and safety, cost-effectiveness and budget impact, organisational and social aspects, and ethical and legal issues. The information is collected and presented in a systematic, unbiased and transparent manner.



HTA criteria

Internal criteria

HTA Committee

1. **Unmet medical need**
2. **Clinical value (based on disease severity, burden of disease, safety, tolerability)**
3. **Therapeutic added value vs current therapies**
4. **Clinical trials' validity**
5. **Cost-effectiveness**

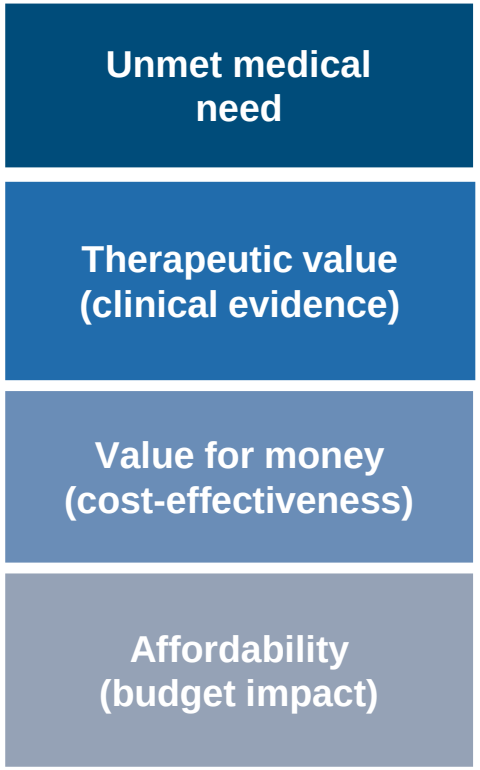
EUnetHTA evaluation will be taken into account

Negotiation Committee

6. Budget impact and reimbursed price

Final Report (Appraisal)

Four key questions for decision-makers



1. Agree there is an **unmet therapeutic need**

2. Agree that the product offers **added therapeutic value** (clinical evidence demonstrates that a product meets unmet medical need better than currently available alternatives)

3. Agree that the product offers good **value for money**
(i.e. it is cost-effective)

4. Agree that the product is **affordable**
(i.e. it has a favorable budget impact)

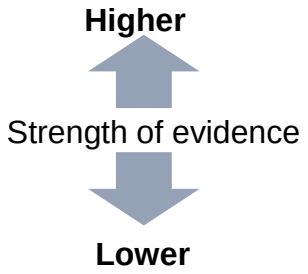
Challenge 1: Data collection

Epidemiology & market size
- ✓ Eligible population
- ✓ Current interventions
- ✓ Uptake of new intervention and market effects

Resource use
- ✓ Physician visits
- ✓ Hospitalizations/Lab & imaging tests
- ✓ Adverse events management

Effectiveness
- ✓ Life years gained
- ✓ Deaths avoided
- ✓ QALYs
- ✓ .....

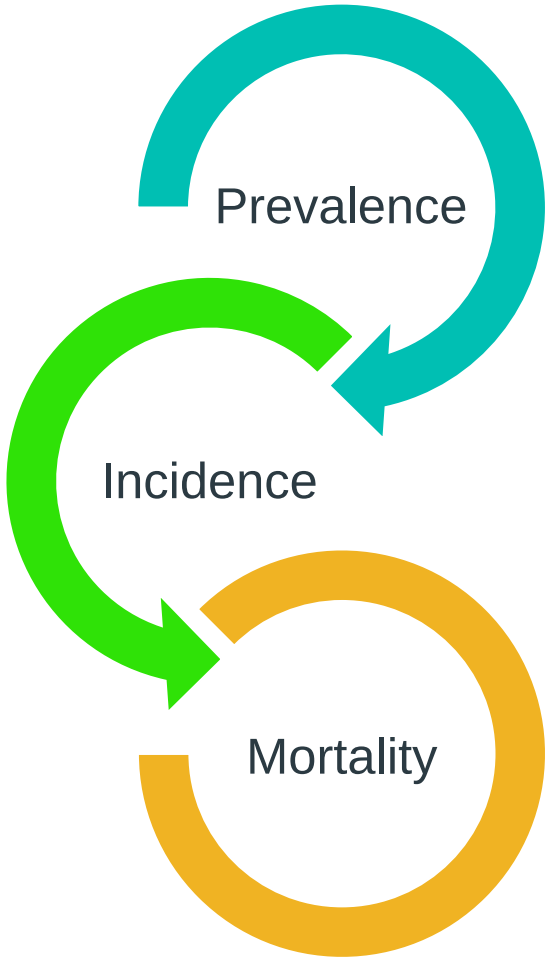
Cost data
- ✓ Physicians visit
- ✓ Lab and imaging test
- ✓ Adverse event
- ✓ .....



Sources of efficacy data	Sources of cost data
Clinical trials	National estimates
Observational studies	Hospitals or institutions
Insurance databases	Delphi panel
Registries	Expert opinion
Market research/ Surveys	
Unpublished data	

Input parameters drive conclusion!!!

The role of epidemiology data in HTA



- Define HTA priorities
- Support unmet medical need
- Burden of disease
- Assessment of cost-effectiveness results
- Estimation of budget impact
- Negotiation

Challenge 2: Clinical efficacy

Direct comparison

- RCTs
- RWE

Indirect comparison:

- ITC: compare alternative treatments in different subpopulations
- MAIC (matching-adjusted indirect comparisons): population-adjusted indirect comparison method that can be used to estimate the relative efficacy of treatment. This alternative ITC approach involves comparing treatments using information from compatible studies and adjusting for differences between their populations

Appropriate comparator

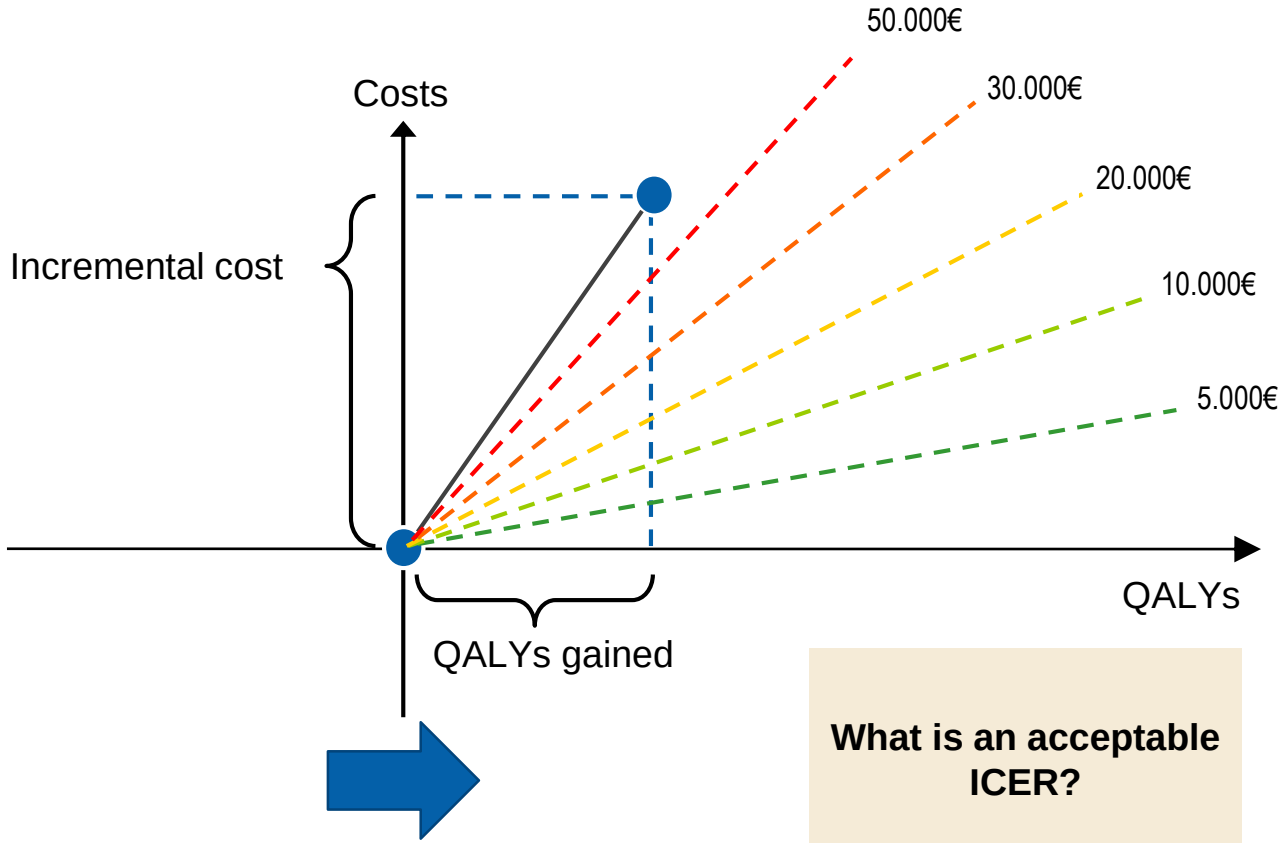
Clinical challenges

- H2H data vs. relevant comparator is missing
- Comparator may vary across countries
- Lack of Real World Evidence/ Issues in dealing with subgroup data
- Limited clinical and PRO/HRQol evidence
- Lack of statistical power

Challenge 3: Cost-effectiveness

- ICER is Judged Relative to a Threshold of Acceptability
- If the ICER is within an acceptable range (threshold) defined by the healthcare payer/provider, then the treatment is likely to be accepted

When drug A has higher treatment costs and higher outcomes than that of drug B, the decision is based on the ICER

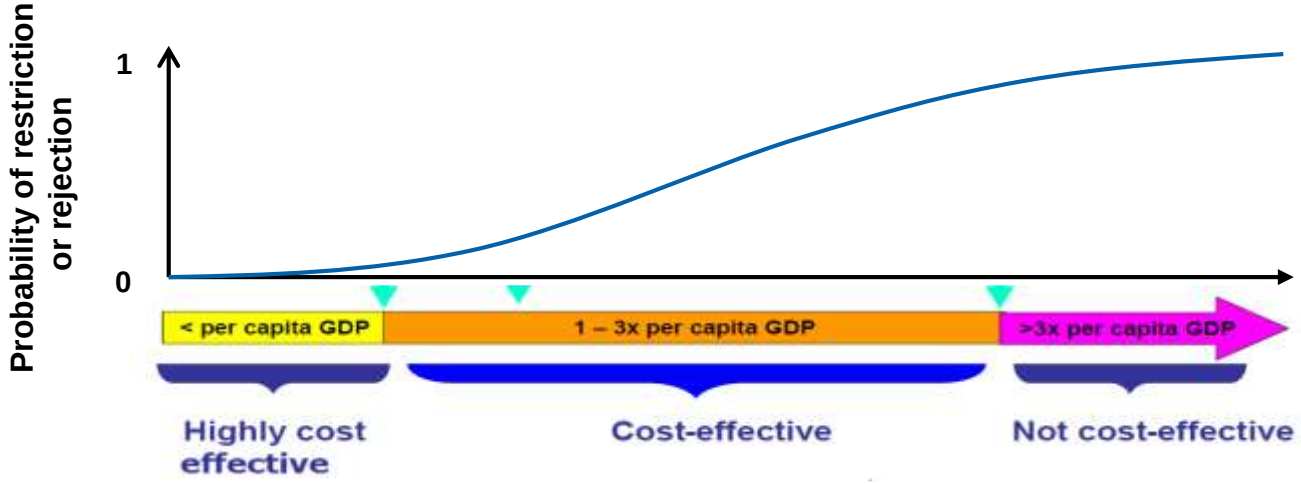


If the ICER is within an acceptable range (threshold) defined by the healthcare payer/provider, then the treatment is likely to be accepted

What is an acceptable ICER?

Lack of ICER threshold in Greece

- WHO definition

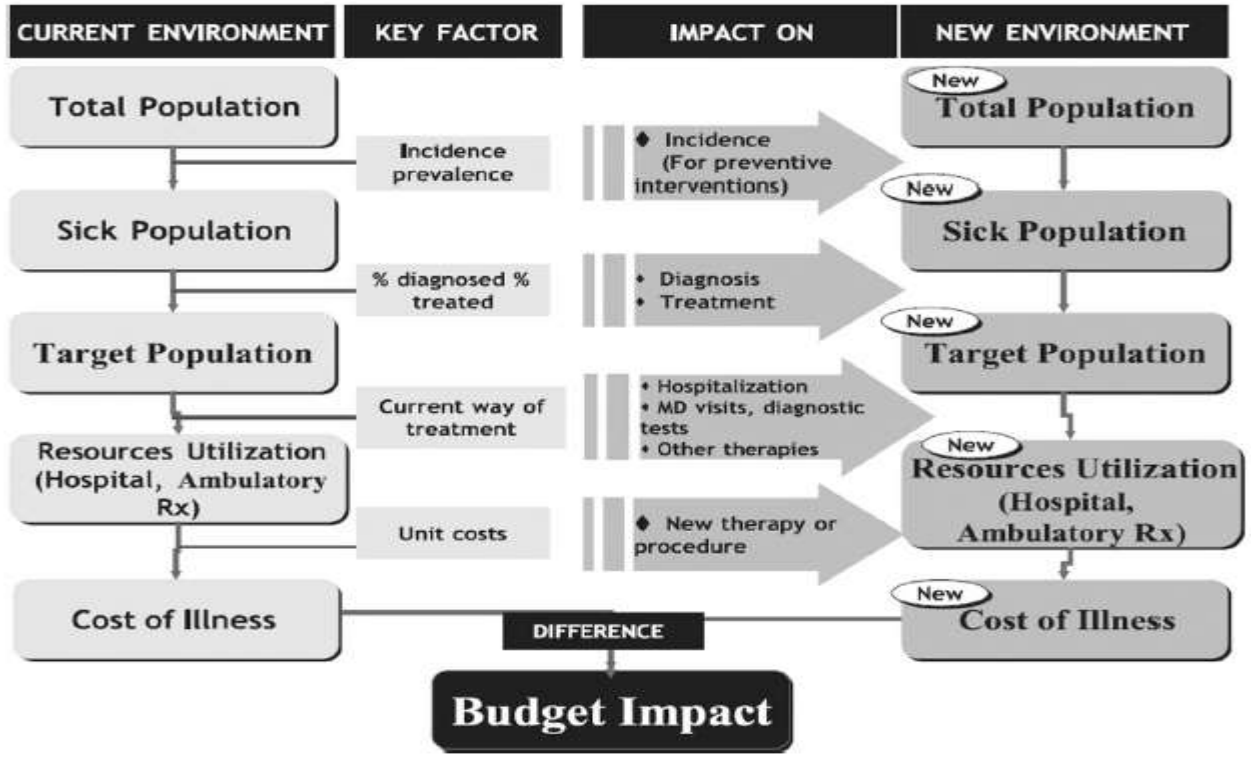


- National Institute for Health and Care Excellence (NICE, UK): Products with ICERs of ~ £ 20,000/QALY – £ 30,000/QALY are more likely to be reimbursed
- US: Products with ICERs below than USD 50,000/QALY are relatively cost-effective, and those with ICERs greater than USD 150,000/QALY suggests against adopting an intervention

HTA & Access challenges

- Uncertainties in cost effective modelling (ICER threshold?)
- Selection of not appropriate comparator
- Comparisons derived via NMA
- Poor value for money

Challenge 4: Budget Impact



Reimbursement & Negotiation challenges

- Local costs are not always available
- Target population definition
- Price used
- High budget impact

Building an HTA dossier is a strategy, not a process

Preparation

- Understand stakeholder's needs
  - patients, payers, providers
- Consider target product profile
- Determine evidence needs
  - what outcomes/endpoints are most important?
- Determine best way to obtain evidence
  - data sources
  - study methods

HTA requirements

1. EPIDEMIOLOGY
2. DISEASE MANAGEMENT
3. DISEASE BURDEN
4. UNMET MEDICAL NEED
5. CLINICAL EVIDENCE
6. ECONOMIC EVALUATION

Evidence

GREEK EPI DATA

COST OF ILLNESS STUDY

QUALITATIVE STUDY

RWE DATA

COST-EFFECTIVENESS & BUDGET IMPACT STUDIES

Thank you